PRESS RELEASE
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New data shows tiratricol (Emcitate®) treatment in patients with MCT8 deficiency is associated with survival benefits

- Abstract by F. van der Most et al. published ahead of the 46th Annual Meeting of the European Thyroid Association, to be held in Athens, Greece, on September 7-10, 2024.
- An international real-world cohort study included data from 228 patients collected from 173 sites in 48 countries.
- Treatment with the investigational drug tiratricol (Emcitate®) in pediatric and adult patients with MCT8 deficiency is associated with an approximately three times lower risk of mortality. This corroborates previous findings indicating that tiratricol sustainably alleviated key clinical features resulting from peripheral thyrotoxicosis.

Stockholm, Sweden, August 21, 2024. Egetis Therapeutics AB (publ) ("**Egetis**" or the "**Company**") (Nasdaq Stockholm: EGTX), today announced the content of an abstract by Dr Floor van der Most and co-authors, Erasmus Medical Center, Rotterdam, The Netherlands, published ahead of the 46th Annual Meeting of the European Thyroid Association, to be held in Athens, Greece, on September 7-10, 2024. In the Abstract, treatment with the investigational drug tiratricol (Emcitate®) in paediatric and adult patients with MCT8 deficiency is associated with an approximately three times lower risk of mortality compared to MCT8 deficiency patients not treated with tiratricol.

This retrospective international real-world cohort study investigated the effects of tiratricol on all-cause mortality in patients with MCT8 deficiency. Genetic, clinical, biochemical and treatment data were collected from 173 sites in 48 countries through an international consortium on MCT8 deficiency, including patients that were in the Triac Trial I, a cohort of patients being part of the tiratricol managed access program, and published cases in the literature. In total 228 patients were included in the study. Baseline characteristics between tiratricol-treated and untreated patients were similar, except for untreated patients residing less often in Western countries (57 vs 78 %). Tiratricol-treated patients had an approximately three times lower risk of all-cause mortality (Hazard Ratio= 0.28, 95% Confidence Interval= 0.09–0.91, p-value <0.05). No other baseline characteristics did significantly affect survival or the effect of tiratricol treatment.

Nicklas Westerholm, CEO, Egetis Therapeutics, commented: *"MCT8 deficiency is a detrimental condition with a median life expectancy of 35 years, significant unmet medical need and no approved treatment. It has already been shown that treatment with tiratricol alleviates thyrotoxicosis by normalizing T3 concentrations and is accompanied by the expected improvement in clinical variables as well as improvements in MCT8 deficiency related caregiver reported outcomes, which formed the basis for the already submitted Marketing Authorisation Application (MAA) in EU. These new encouraging data on the effects of tiratricol treatment on survival, with an estimated three times lower risk of mortality, further shows the clinical importance of treating the chronic thyrotoxicosis present in all patients with MCT8 deficiency. This was also recently confirmed in the new ETA guidelines, which recommends tiratricol (Emcitate®) as long-term therapy for all patients with MCT8 deficiency. The mortality data adds to the totality of evidence on the benefits of the investigational drug, and we aim to share these new survival findings both with the EMA and the FDA. We are excited about hopefully being able to provide the first approved treatment for patients with MCT8 deficiency."*

Results from the study, will be presented as an oral presentation by Dr Floor van der Most on September 8, 2024, at the 46th Annual Meeting of the European Thyroid Association, in Athens, Greece.

Link to the Abstract: Van der Most, F. et al. *T3 analogue Triiodothyroacetic acid (Triac) treatment and survival in MCT8 deficiency: an international real-world cohort study*
Link to the conference program: *https://apps.m-anage.com/eta2024/en-GB/pag/presentation/673248*

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For further information about MCT8 deficiency, please see www.mct8deficiency.com

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About Egetis Therapeutics

Egetis Therapeutics is an innovative and integrated pharmaceutical company, focusing on projects in late-stage development for commercialization for treatments of serious diseases with significant unmet medical needs in the orphan drug segment.

The Company's lead drug candidate Emcitate® (tiratricol) is under development for the treatment of patients with monocarboxylate transporter 8 (MCT8) deficiency, a highly debilitating rare disease with no available treatment. In previous studies (Triac Trial I and a long-term real-life study) *Emcitate* has shown highly significant and clinically relevant results on serum thyroid hormone T3 levels and secondary clinical endpoints. Egetis submitted a marketing authorisation application (MAA) for *Emcitate* to the European Medicines Agency (EMA) in October 2023.

After a dialogue with the FDA, Egetis is conducting a randomized, placebo-controlled pivotal study in 16 evaluable patients to verify the results on T3 levels seen in previous clinical trials and publications. Egetis will update the market as soon as recruitment has been completed and at that point inform about the timing of availability of top-line results, and the expected timing of the subsequent NDA filing.

Emcitate holds Orphan Drug Designation (ODD) for MCT8 deficiency and resistance to thyroid hormone type beta (RTH-beta) in the US and the EU. MCT8 deficiency and RTH-beta are two distinct indications, with no overlap in patient populations. *Emcitate* has been granted Rare Pediatric Disease Designation (RPDD) which gives Egetis the opportunity to receive a Priority Review Voucher (PRV) in the US, after approval. This voucher can be transferred or sold to another sponsor.

The drug candidate Aladote® (calmangafodipir) is a first in class drug candidate developed to reduce the risk of acute liver injury associated with paracetamol (acetaminophen) overdose. A proof of principle study has been successfully completed. The design of a pivotal Phase IIb/III study (Albatross), with the purpose of applying for market approval in the US and Europe, has been finalized following interactions with the FDA, EMA and MHRA. The study start has been postponed until *Emcitate* marketing authorization submissions for MCT8 deficiency have been completed. *Aladote* has been granted ODD in the US and in the EU.

Egetis Therapeutics (STO: EGTX) is listed on the Nasdaq Stockholm main market. For more information, see www.egetis.com

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Attachments

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